

# GROWING GLOBAL INTEREST IN OMAPRO™

As the positive OMAPRO™ data from clinical trials have become known, the demand for OMAPRO™ under a compassionate use/expanded access or a similar method has increased.

Meeting the needs of patients is a primary concern for ChemGenex, and the establishment of compassionate use access to OMAPRO™ has been a priority. Now established, this process is providing OMAPRO™ access to physicians and their patients around the world.

Under this program, ChemGenex has provided OMAPRO™ to patients from many regions across the globe, including: Australia, North America, South America, Europe and Africa.

"Omacetaxine is a valuable option for the treatment of patients with CML, particularly those in two categories where we do not have any available treatment options. These are patients who have failed at least two prior tyrosine kinase inhibitors and those who have a mutation T315I, since we know that with this mutation none of the available tyrosine kinase inhibitors has activity. These results correspond well with the known activity of this compound in CML."

Dr. Jorge Cortes, MD, Professor of Medicine and Deputy Chair in the Department of Leukemia at The University of Texas, MD Anderson Cancer Center. Cancer Institute

# **KEY MANAGEMENT**

Greg Collier, PhD
Chief Executive Officer and Managing Director
Adam Craig, MD, PhD, MBA
Senior Vice President and Chief Medical Officer

James Campbell, PhD, MBA Chief Financial Officer and Chief Operating Officer Tom DeZao, BA

Senior Vice President and Chief Commercial Officer
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ChemGenex currently trades on the Australian Stock Exchange under the symbol 'CXS,'

Certain statements made herein use forward-looking statements within the meaning of the US Private Securities Litigation Reform Act of 1995. These forward-looking statements involve known and unknown risks and uncertainties which could cause the actual results, performance or achievements of the company to be materially different from those which may be expressed or implied by such statements, nicluding, among others, risks or uncertainties associated with the development of the company's technology, the ability to excessfully market products in the clinical pipeline, the ability to advance promising therapeuts through clinical thats, the ability to exclusions out ruly integrated technologies, the ability to exist into additional collaborations and strategic aliances and expand current collaborations.



New therapies for hematological malignancies



#### COMPANY PROFILE

ChemGenex is a biopharmaceutical company focused on developing small molecules with novel mechanisms of action to treat hematologic malignancies where there are significant unmet medical needs.

## **LEAD DRUG CANDIDATE**

The company's lead investigational agent is OMAPRO™ (omacetaxine mepesuccinate), a subcutaneous injection for the treatment of patients with chronic myeloid leukemia (CML).

#### **REGULATORY STATUS**

OMAPRO™ is an investigational agent and does not have marketing authorization in the US or EU.

A Marketing Authorisation Application (MAA) is under review by the European Medicines Agency for CML patients who have failed imatinib and have the Bcr-Abl T315I mutation.

A new NDA, for omacetaxine in CML patients who have failed multiple tyrosine kinase inhibitors is in preparation for submission to the FDA.

### **COMMERICAL STRATEGY**

ChemGenex intends to commercialize omacetaxine itself in North America and has established a corporate alliance with Hospira to develop and commercialize omacetaxine in Europe, the Middle East and Africa. The company is seeking to establish similar commercial partnerships throughout the rest of the world.

# **DEVELOPMENT PIPELINE**

The company is focusing the majority of its efforts on the evaluation of OMAPRO $^{\text{TM}}$  in CML and other hematological disorders.

# **CURRENT CLINICAL TRIALS**

The company's pivotal clinical trials, which have completed enrollment, were designed to elucidate the therapeutic profile of omacetaxine (commercial name OMAPROTM) in patients who were resistant to treatment with two or more approved TKIs (Study 203) and in patients who had failed treatment with imatinib and carried the T315l kinase domain mutation (Study 202).

#### STUDY CML-202

Registration directed Phase 2 pivotal study in T315I+ CML patients

Enrollment: Complete (103 patients)

#### STUDY CML-203

Registration directed Phase 2 study in CML patients with multi-TKI failure

Enrollment: Complete (100 patients)

# **CLINICAL TRIALS PLANNED**

Pre-clinical research has shown that OMAPRO™ can kill human CML stem cells in vitro and peripheral leukemic cells, raising the possibility that OMAPRO™ could provide a unique avenue of treatment for CML and other leukemias. Consequently, future trials will evaluate the potential synergistic activity of omacetaxine in combination with TKIs.

Published studies also indicate that omacetaxine has activity in other hematologic malignancies. In the future, ChemGenex plans to characterize the potential benefits of OMAPRO™ in acute myeloid leukemia (AML) and myelodysplastic syndrome (MDS).

## OMAPRO™ MODE OF ACTION

Protein synthesis is markedly up-regulated in malignant cells and high levels of many 'short lived proteins' (oncoproteins). These oncoproteins promote cell division, suppress apoptosis and are prevalent in many cancer types including CML.

Drugs that can interfere with the manufacture of these 'short-lived' proteins offer a new and unique way of attacking CML, particularly where TKIs have failed to have their desired effect.

OMAPRO™ works by inhibiting protein translation and is particularly active against a number of short lived proteins that are associated with CML, AML and multiple myeloma.



OMAPRO™ binds to the ribosomal A-cleft (shown above), preventing the translation and elongation of short-lived oncoproteins.